Hemophilia and Other Hereditary Bleeding Disorders

WHAT IS THE PUBLIC HEALTH ISSUE?

Hemophilia is an inherited bleeding disorder that affects 18,000 persons (primarily males) in the United States. The disorder results from deficiencies in blood clotting factors and can lead to spontaneous internal bleeding and bleeding following injuries or surgery. These bleeding episodes can cause severe joint damage, neurological damage, damage to other organ systems involved in the hemorrhage, and, in rare cases, death. Treating the bleeding episodes involves the prompt and proper use of clotting factor concentrates. The health and welfare of hemophilia patients and their families have been significantly affected by the HIV epidemic. As many as 65 percent of persons with hemophilia in the United States were infected with HIV by 1985 as a direct result of receiving contaminated clotting factor concentrates. To avoid future outbreaks of bloodborne infections, a surveillance system was developed to monitor possible bloodborne infections in the hemophilia community as part of a comprehensive prevention program. There are other hereditary bleeding disorders, including von Willebrand disease, which is the most common—found in approximately 1-2 percent of the U.S. population.

WHAT HAS CDC ACCOMPLISHED?

- Established and coordinated a network of hemophilia treatment centers (HTCs) to promote the management, treatment, and prevention of complications experienced by persons with hemophilia and other hereditary bleeding disorders.
- Developed a system to monitor blood safety and conduct health-care-outcome research by integrating these activities into the HTC network.
- Collaborated with the National Hemophilia Foundation in developing a national health campaign to encourage individuals to adopt healthy behaviors and participate in the HTC network. Encouraged the national foundation to work closely with local community-based organizations to deliver the prevention messages of the campaign.
- Demonstrated the effectiveness of the HTC network through improved outcomes, such as decreased mortality and hospitalization rates.
- Initiated studies to evaluate measures of preventing joint disease and determined the prevalence of bloodstream infections associated with the use of central venous access devices.
- Established a reference laboratory to diagnose rare bleeding disorders, and initiated studies to improve laboratory methods used to diagnose bleeding disorders.

WHAT ARE THE NEXT STEPS?

- Expand the surveillance system to include children between the ages of 0 and 2 years.
- Develop methods to predict the predisposition of patients to develop inhibitors against factor concentrates.
- Continue investigation of new pathogens that may threaten the blood supply including West Nile Virus.
- Continue monitoring individuals to determine whether their joint disease has progressed, and continue studying potential interventions to prevent such progression.
- Initiate a comparative study of joint range of motion in persons that do not have a bleeding disorder.
- Develop methods to measure the economic and quality-of-life benefits associated with interventions designed to reduce the risk of adverse health outcomes associated with bleeding and clotting disorders.
- Explore opportunities to use surveillance systems and data for broader research efforts.
- Develop methods to predict inhibitors as well as patients' responses to gene therapy trials by evaluating genetic markers.
- Expand the outreach initiatives so that under-served groups with hemophilia have better access to care and prevention services.

For information on this and other CDC and ATSDR programs, visit www.cdc.gov/programs. December 2004